

ABSTRACT OF THE DISCLOSURE

The present invention concerns compounds, compositions, and methods for the study, diagnosis, and treatment of diseases and conditions associated with polyglutamine repeat (polyQ) allelic variants that respond to the modulation of gene expression and/or activity.

- 5 The present invention also concerns compounds, compositions, and methods relating to diseases and conditions associated with polyglutamine repeat (polyQ) allelic variants that respond to the modulation of expression and/or activity of genes involved in polyQ repeat gene expression pathways or other cellular processes that mediate the maintenance or development of polyQ repeat diseases and conditions such as Huntington disease and related
- 10 conditions such as progressive chorea, rigidity, dementia, and seizures, spinocerebellar ataxia, spinal and bulbar muscular dystrophy (SBMA), dentatorubropallidoluysian atrophy (DRPLA), and any other diseases or conditions that are related to or will respond to the levels of a repeat expansion (RE) protein in a cell or tissue, alone or in combination with other therapies. Specifically, the invention relates to small nucleic acid molecules, such as short
- 15 interfering nucleic acid (siNA), short interfering RNA (siRNA), double-stranded RNA (dsRNA), micro-RNA (miRNA), and short hairpin RNA (shRNA) molecules capable of mediating RNA interference (RNAi) against the expression disease related genes or alleles having polyQ repeat sequences.